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**Optimization of a gene therapy for inherited erythromelalgia in iPSC-derived neurons**

**Grant Award Details**

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Optimization of a gene therapy for inherited erythromelalgia in iPSC-derived neurons

**Grant Type:** Quest - Discovery Stage Research Projects

**Grant Number:** DISC2-13013

**Investigator:**

<b>Name:</b>	Ana Moreno
<b>Institution:</b>	Navega Therapeutics
<b>Type:</b>	PI

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**Disease Focus:** Neurological Disorders

**Human Stem Cell Use:** iPS Cell

**Award Value:** \$1,157,313

**Status:** Pre-Active

**Grant Application Details**

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**Application Title:** Optimization of a gene therapy for inherited erythromelalgia in iPSC-derived neurons

**Public Abstract:****Research Objective**

The goal of this grant is to develop a gene therapy for a rare painful disorder, Inherited Erythromelalgia (IEM).

**Impact**

There are currently no FDA approved drugs for IEM, which is caused by a gain-of-function mutation in a sodium channel, Nav1.7. We propose epigenetic repression of Nav1.7 to provide a cure for IEM.

**Major Proposed Activities**

- We will characterize the lead gene therapy candidate that will move into IND-enabling studies.
- We will determine the efficacy of this lead candidate in a clinically relevant human cell population (patients with Inherited Erythromelalgia).
- We will perform dose range studies to provide preliminary identification of the target organs of toxicity as well as to select doses for future definitive toxicology studies in non-human primates.
- We will request a FDA meeting.

**Statement of Benefit to California:**

It is estimated that 50 million Americans suffer from chronic pain, with patients relying mostly on opioids. In California, an estimated 45% of drug overdose deaths involved opioids in 2018. We are in dire need of new treatments for chronic pain. Although our first indication will be a rare painful condition, our gene therapy could potentially benefit other individuals with intractable painful conditions, as the gene we are targeting is involved in pain transmission and in many pain conditions.

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